Genome editing for causation and reversion of MPN-associated mutations in human hematopoietic stem cells

Grant Award Details

Genome editing for causation and reversion of MPN-associated mutations in human hematopoietic stem cells

Grant Type: Inception - Discovery Stage Research Projects

Grant Number: DISC1-08776

Project Objective: To create CRISPR/Cas9 reagents that are capable of generating and reverting MPN associated mutations in various cell types, including primary human hematopoietic stem cells (HSCs).

Investigator:

Name:	Jacob Corn
Institution:	University of California, Berkeley
Type:	PI

Disease Focus: Blood Cancer, Cancer

Human Stem Cell Use: Adult Stem Cell

Award Value: $235,800

Status: Active

Grant Application Details

Application Title: Genome editing for causation and reversion of MPN-associated mutations in human hematopoietic stem cells

Public Abstract:

Research Objective

Use gene editing to create tools for the study of mechanisms by which patient-observed mutations lead to myeloproliferative neoplasms.

Impact

Editing reagents will yield new insight into how acquired MPN-associated mutations cause disease by overproduction of various cell types and pave the way for gene editing therapies to reverse MPNs.

Major Proposed Activities

- Develop tools to enable the study of MPN-associated mutations at endogenous loci.
- Create isogenic human cell lines harboring MPN-associated mutations and introduce mutations to primary human CD34+ HSCs.

Statement of Benefit to California:

The research described in this proposal will both yield new information to the MPN family of hematopoietic disorders and broadly enable the creation and reversion of mutations in hematopoietic stem cells. New understanding of MPNs could lead to improved drugs to treat these disorders. Methods to perform gene editing in hematopoietic stem cells could be transformative for the treatment of inherited and acquired blood diseases, potentially allowing gene editing therapies to cure such disorders.
